Agenda (I)

Set up
9 – 9:30

- **Galen/Atlantica opening:** Introductions, objectives, ground rules
- **Peter Marks (FDA):** Pursuing the long tail of rare diseases, FDA objectives
- **Mike Lehmicke (ARM):** Opening remarks

Problem definition & impact
9:30-10:45

Phil Kurs (FDA)
- Statutory considerations shaping the Designation Program for Platform Technologies

Fyodor Urnov (Innovative Genomics Institute, UC Berkeley)
- Development approaches and risk-benefit for an ‘n of 1’ therapy

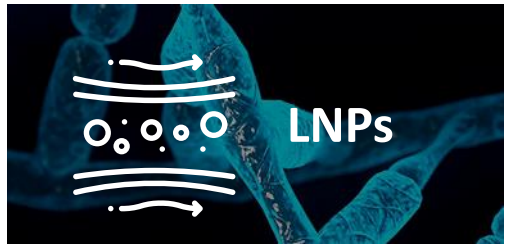
Galen/Atlantica synthesis of opportunities from pre-meeting discussions
- Introducing building blocks
- Vehicles to deliver building blocks, and evidentiary requirements

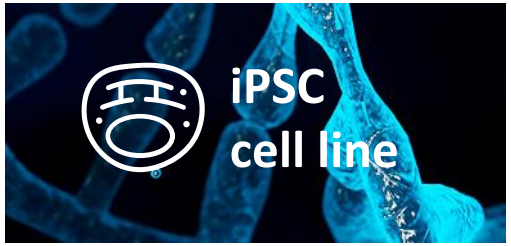
What are the limitations of cell and gene therapy without building blocks? What is the role of every stakeholder in promoting standardization?
- *Group discussion*

Break

Consideration of building blocks
11:00 – 1:00

Technology-specific breakout groups to populate building block framework (distribute)
- ***Describe****:* What element of development, manufacture or delivery can be re-used across programs?
- ***Defend:*** What is the case for this element as a building block? Contrarian views?
- ***Define:*** Define What are the measurable parameters that define that building block?
- ***Bound:*** What specific elements (e.g., manufacturing) of a building block must be fixed across applications?
- ***Disseminate:*** What is the vehicle for the developer or industry to reference a building block?
- ***Value:*** What steps in development can be omitted or done more efficiently? What is the value?





Agenda (II)

Lunch (1:00 pm)

Case study outcomes, implications
1:45 – 3:30

Individual case study teams present findings back to group

- Developer perspectives
- FDA evidence requirements
- Discussion

Common themes across cases studies

- What defines a robust building block?
- On what aspects of drug development should companies compete, and where are pre-competitive approaches needed?
- What do developers need to do differently to introduce and validate building blocks? What does the FDA need to do differently?

Break (if needed)

Next steps and closing
3:30 – 4:00

- **Peter Marks:** Closing reflections and take-aways
- **Galen/Atlantica:** Roadmap for progress
- **Close**